A Phase 1/2, First-in-Human, Open-Label, Dose-Escalation Study of the Safety and Pharmacodynamic Activity of Gene Therapy for Congenital Adrenal Hyperplasia through Administration of an Adeno-associated Virus (AAV) Serotype 5-Based Recombinant Vector Encoding the Human CYP21A2 Gene

Status: Recruiting

Eligibility Criteria

Age: 18 years and over

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

- adults with classic Congenital Adrenal Hyperplasia (CAH) - on stable oral hydrocortisone (HC) regimen as the only glucocorticoid (GC) maintenance therapy - no prior gene therapy or AAV-mediated therapy

Exclusion Criteria:

- positive for anti-AAV5 (Adeno-Associated Virus Type 5) antibodies - history of adrenalectomy and/or significant liver disease - women who are pregnant

Conditions & Interventions

Conditions:
Diabetes & Endocrine
Keywords:
Congenital Adrenal Hyperplasia

More Information

Description: This is a study designed to evaluate the safety, tolerability, and efficacy of a one-time gene therapy (BBP-631) for adult patients diagnosed with classic congenital adrenal hyperplasia (CAH). The goal of gene therapy for CAH is to give the body a functioning CYP21A2 gene using a vector (an agent used to deliver a gene into the body). Having a functioning CYP21A2 gene in the adrenal gland may allow the body to naturally produce its own cortisol and aldosterone. The study treatment and follow-up lasts 1 year with a long-term follow-up of 4 more years.
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